

(19) World Intellectual Property Organization  
International Bureau



(43) International Publication Date  
23 October 2003 (23.10.2003)

PCT

(10) International Publication Number  
**WO 03/087368 A2**

- (51) International Patent Classification<sup>7</sup>: **C12N 15/11**,  
A61K 48/00, 31/713, 9/08, C12Q 1/68, C12N 15/12,  
C07K 14/47, 16/18, G01N 33/50, 33/68, A01K 67/027
- (21) International Application Number: **PCT/EP03/04003**
- (22) International Filing Date: **16 April 2003 (16.04.2003)**
- (25) Filing Language: **English**
- (26) Publication Language: **English**
- (30) Priority Data:  
02008761.5 **18 April 2002 (18.04.2002)** **EP**  
60/431,173 **5 December 2002 (05.12.2002)** **US**
- (71) Applicant (*for all designated States except US*):  
**LYNKEUS BIO TECH GMBH** [DE/DE]; Friedrich-  
Bergius-Ring 15, 97076 Würzburg (DE).
- (72) Inventors; and
- (75) Inventors/Applicants (*for US only*): **DRUMM, Karina**  
[DE/DE]; Innerer Graben 12, 97070 Würzburg (DE).  
**SCHLÖR, Stefan, Hubert** [DE/DE]; Alfred-Delp-Str.  
9c, 97828 Marktheidenfeld (DE). **GÖHRING, Frank**  
[DE/DE]; Robert-Kirchhoff-strasse 35, 97076 Würzburg  
(DE).
- (74) Agent: **KNAUTHE EGGERS**; Postfach 31 01 40, 80102  
München (DE).
- (81) Designated States (*national*): AE, AG, AL, AM, AT, AU,  
AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU,  
CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GE, GH,  
GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC,  
LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW,  
MX, MZ, NO, NZ, OM, PH, PL, PT, RO, RU, SC, SD, SE,  
SG, SK, SL, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ,  
VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (*regional*): ARIPO patent (GH, GM,  
KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW),  
Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM),  
European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE,  
ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO,  
SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM,  
GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).
- Published:**  
— *without international search report and to be republished  
upon receipt of that report*
- For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.*

(54) Title: MEANS AND METHODS FOR THE SPECIFIC MODULATION OF TARGET GENES IN THE CNS AND THE EYE AND METHODS FOR THEIR IDENTIFICATION

(57) Abstract: Provided are methods for the treatment of disorders of the central nervous system (CNS) and the eye. In particular, use of compositions comprising a compound capable of modulating a target gene or gene product is described for the preparation of a pharmaceutical composition for the treatment of disorders of the CNS and/or the eye, wherein the composition is designed to be administered outside the blood-CNS and the blood-retina barriers. Furthermore, methods are provided for identifying and obtaining nucleic acid molecules encoding polypeptides involved in CNS disorders or of the eye, methods for diagnosing said disorders as well as transgenic animal deficient in the expression of target genes identified in accordance with the described method. In addition, methods of identifying and isolating drugs that are particularly useful for the treatment of disorders related to the CNS and/or the eye are disclosed.